

-continued

(i) SEQUENCE CHARACTERISTICS:

- (A) LENGTH: 145 base pairs
- (B) TYPE: nucleic acid
- (C) STRANDEDNESS: single
- (D) TOPOLOGY: linear

(ii) MOLECULE TYPE: DNA (genomic)

(xi) SEQUENCE DESCRIPTION: SEQ ID NO: 1:

TTGGCCACTC CCTCTCTGCG CGCTCGCTCG CTCACTGAGG CCGGGCGACC AAAGGTGCC	60
CGACGCCCGG GCTTTGCCCG GGCGGCCTCA GTGAGCGAGC GAGCGCGCAG AGAGGGAGTG	120
GCCAACCTCCA TCACTAGGGG TTCCT	145

(2) INFORMATION FOR SEQ ID NO: 2:

(i) SEQUENCE CHARACTERISTICS:

- (A) LENGTH: 225 base pairs
- (B) TYPE: nucleic acid
- (C) STRANDEDNESS: single
- (D) TOPOLOGY: linear

(ii) MOLECULE TYPE: DNA (genomic)

(xi) SEQUENCE DESCRIPTION: SEQ ID NO: 2:

TTTTAGCGGG CTTTTTCCC GCCTTATGCA AATGGGCAGC CATTTAACGT GTTTTACTAT	60
AATTTTATTG GTTAGTTTG TAACGGTTAA AATGGGCAGA GCGTAGGCGG GGACTACAGT	120
ATATATAAGCA CGGTACTGCC GCAGCTCTTT CTTCTGGGC TGCTTTTCC TGGACTTCT	180
TGCTGTTTT TGTGAGCTAA CTAACAGGTA TTTATACTAC TTGTT	225

What is claimed:

1. An expression vector for site-specific integration and cell-specific gene expression comprising two inverted terminal repeats of adeno-associated virus 2 and at least one cassette comprising a promoter capable of effecting cell-specific expression wherein said promoter is operably linked to a heterologous gene, and wherein said cassette resides between said inverted terminal repeats.
2. The vector of claim 1 wherein each of said inverted terminal repeats comprises the nucleotides of SEQ ID NO:1.
3. The vector of claim 1 wherein each of said inverted terminal repeats comprises nucleotides 1 to 125 of SEQ ID NO:1.
4. The vector of claim 1 wherein said heterologous gene encodes a biologically functional protein.
5. The vector of claim 1 wherein said heterologous gene encodes a non-biologically functional protein.
6. The vector of claim 1 wherein said heterologous gene encodes an antisense RNA.
7. The vector of claim 1 wherein said heterologous gene is selected from the group consisting of a gene encoding α -globin, β -globin, γ -globin, granulocyte macrophage-colony stimulating factor (GM-CSF), tumor necrosis factor

35 (TNF), any one of interleukins 1-11, neomycin resistance, luciferase, adenine phosphoribosyl transferase (APRT), retinoblastoma, insulin, mast cell growth factor, p53, adenosine deaminase.

40 8. The vector of claim 1 wherein said heterologous gene encodes P-glycoprotein.

9. The vector of claim 6 wherein said antisense RNA is complementary to a segment of the DNA or RNA encoding α -globin.

45 10. The vector of claim 1 wherein said vector is AAV-B19-mdr.

11. A host cell transfected by the vector of any one of claims 1-10.

50 12. The host cell of claim 11 wherein said cell is a hematopoietic stem or hematopoietic progenitor cell.

13. A virion comprising the vector of any one of claims 1-9.

55 14. A host cell infected by the virion of claim 13.

15. The host cell of claim 14 wherein said cell is a hematopoietic stem or progenitor cell.

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